

ABSTRACT

5 Novel genes and vectors exhibiting increased expression and novel splicing patterns
are disclosed. The gene can comprise one or more consensus or near consensus splice sites
which have been corrected. The gene can alternatively or additionally comprise one or more
introns within coding or noncoding sequences. The gene can still further comprise modified
10 5' and/or 3' untranslated regions optimized to provide high levels and duration of tissue-
specific expression. In one embodiment, the gene comprises the coding region of a full-
length Factor VIII gene modified by adding an intron within the portion of the gene encoding
the β -domain, so that the gene is expressed as a β -domain deleted Factor VIII protein. The
novel Factor VIII gene can also be modified to correct one or more consensus or near
consensus splice sites within or outside of the coding region.